# Development of a Chondrogenic Drug Candidate Targeting Resident Mesenchymal Stem Cells for the Treatment of Osteoarthritis

## Grant Award Details

Development of a Chondrogenic Drug Candidate Targeting Resident Mesenchymal Stem Cells for the Treatment of Osteoarthritis

**Grant Type:** Late Stage Preclinical Projects

**Grant Number:** CLIN1-08309

**Project Objective:** To develop a small molecule treatment for osteoarthritis injected intra-articularly to enhance MSC chondrogenic differentiation in vivo. This award is running in parallel with the team's PC1 award. This CLIN1 award funds the GMP manufacturing of the drug and the conduct of the IND-enabling toxicology studies to complete the IND package and file an IND with the FDA for a Phase 1 trial.

**Investigator:**

| | |
|---|---|
| **Name:** | Peter Schultz |
| **Institution:** | California Institute for Biomedical Research |
| **Type:** | PI |

**Disease Focus:** Bone or Cartilage Disease

**Award Value:** $1,667,832

**Status:** Closed

## Progress Reports

**Reporting Period:** Operational Milestone (OM) #1

**View Report**

**Reporting Period:** Final Operational Milestone #2

**View Report**

## Grant Application Details

**Application Title:** Development of a Chondrogenic Drug Candidate Targeting Resident Mesenchymal Stem Cells for the Treatment of Osteoarthritis

**Public Abstract:**

**Therapeutic Candidate or Device**

The therapeutic candidate is a drug-like small molecule that promotes cartilage resident mesenchymal stem cell differentiation into chondrocytes.

**Indication**

Osteoarthritis and cartilage injury

**Therapeutic Mechanism**

The therapeutic candidate, through intra-articular administration, promotes cartilage resident mesenchymal stem cell differentiation into chondrocytes. The newly formed chondrocytes replace the dead chondrocytes, synthesize and secret extracellular matrix proteins, which leads to the repair of damaged cartilage in OA patients or following traumatic injury.

**Unmet Medical Need**

Current therapeutic options for OA are limited to pain or symptom-modifying drugs and joint replacement surgery; no disease-modifying drugs are approved for clinical use. The therapeutic candidate, if successful, will be the first-in-class regenerative medicine for OA and cartilage injury.

**Project Objective**

IND filing and initiating Phase 1 clinical trial

**Major Proposed Activities**

- IND document preparation and filing
- GLP toxicology and safety profiling of the therapeutic candidate
- Non-GLP determination of maximum tolerated doses upon local administration

**Statement of Benefit to California:**

Osteoarthritis (OA) is the most prevalent musculoskeletal disease and globally the 4th leading cause of Years Lost to Disease (YLD). The annual economic impact of arthritis in the U.S. is estimated at over $100 billion. No disease-modifying OA drugs are approved for clinical use. Clearly the development of a new disease-modifying therapeutic would have a significant impact on the well-being of Californians and reduce the negative economic impact on the state.

---

**Source URL:** https://www.cirm.ca.gov/our-progress/awards/development-chondrogenic-drug-candidate-targeting-resident-mesenchymal-stem